DESCRIPTION provided by applicant A Phase II SBIR program of preclinical development is proposed for the development of an active specific immunotherapeutic called Cellarium® for treatment of glial cell based cancers. Successful completion of this project will confirm therapeutic effect and pre clinical safety of the vaccine in preparation for first in human studies. Upon demonstration of safety and efficacy the Cellarium vaccine will provide a greatly improved targeted treatment for glioma patients for whom current treatments can be debilitating, painful and expensive. Gliomas are widespread deadly diseases for which current therapies are inadequate. In the US gliomas accounted for of the cases of brain and other nervous system cancers diagnosed and associated deaths in Recent studies have shown increasing incidence of glioma in US adults. Gliomas are predominantly treated today with an aggressive three step protocol of surgery chemotherapy and radiation. Despite this regimen patients with Glioblastoma Multiforme GBM, the most common and deadly malignant brain tumor still have a median survival of less than months. Moreover, the estimated cost of treatment for each patient with a malignant brain tumor ranges from $ to several hundred thousand dollars annually. Originally this technology approach was conceived in the laboratory of Dr. David Mooney at the University of Michigan who is now at Harvard University. The Cellarium technology is the result of work at InCytu in both the melanoma and glioma models that has led to new insights which transcend the original discoveries at Michigan and Harvard and have led to a more effective and reliable product. The Cellarium is an in situ therapeutic vaccine which incorporates biological cofactors and patient specific cell lysate attracts dendritic cells DCs exposes them to the tumor specific antigens and then releases them to direct a strong targeted immune response against malignant tissue. Phase II aims are to manufacture Cellarium using equipment and methods purpose designed to yield CNS specific product sized for both the murine and human sized brain and confirm Phase I results of the Cellarium for Glioma in both murine and porcine preclinical models to develop formal Standard operating Procedures needed for submission to the FDA and validate manufacture and QC of the devices then perform a pre GLP study needed for discussions with the FDA to conduct GLP safety and
efficacy study in preparation for the filing of an IND. If Phase II studies are successful, the Cellarium vaccine will continue to be developed through clinical trials with the eventual goal of scaling manufacture in the US and selling as a commercially available specific immunotherapeutic vaccine to treat glioma patients.

PUBLIC HEALTH RELEVANCE: Glioma is a growing public health problem, with deaths in current therapies being expensive andgt $ k with severe side effects and a difficult median survival of &lt months, with very few remissions to treat. A breakthrough immunotherapy called Cellarium showed very promising results in a rat model performed under a Phase I SBIR program. This project will complete preclinical development of the Cellarium glioma therapeutic vaccine in preparation for filing an IND and a first-in-man study.

* Information listed above is at the time of submission. *