### **IUPUI**

## Innovation to Enterprise Showcase & Forum

### EMPHYMAB BIOTECH, MEDICAL THERAPIES FOR EMPHYSEMA

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Industry Sector(s): Healthcare, Pharmaceutical Product Category: Therapeutic antibody

### Opportunity Overview

Emphymab<sup>TM</sup> Biotech was formed to develop and commercialize medical therapies that address serious lung diseases. The founders are scientists and clinicians at Indiana University School of Medicine. Emphymab's lead technology is based on a novel monoclonal antibody that inactivates a newly discovered pathway involved in lung diseases and, thereby, halts progressive loss of lung function associated with emphysema. This technology has the potential to address the huge unmet medical need of patients suffering from chronic obstructive pulmonary disease (COPD) with emphysema, which is the 3rd leading cause of death worldwide.

### Markets & Applications

The US market is comprised of 4.3 million emphysema patients.

Based on our preclinical data, a treatment regimen would consist of 6 total doses (2 doses/month for 3 months). The price per dose is predicted to be \$800, which is based the on current costs to patients for therapeutic antibodies. Therefore, the total available US market is over \$20 billion.

### Competitive Advantage/Value Propositions

Novel therapeutic targets for emphysema, identified through studies of the mechanisms of disease-induced lung destruction, with pending U.S. patent and international patent applications

Ongoing developmental activities, including creation of a humanized monoclonal antibody, will be proposed in our subsequent SBIR phase II grant proposal, which will have a budget total of up to \$1 million over a 2 year period to complete these and other development activities

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### Researcher Biographies

### Brian Johnstone, Ph.D.

Dr. Johnstone, (EmphyMab founder), is currently an Associate Research Professor of Medicine at IU School of Medicine. He is also the Preclinical Director for the Regenerative Medicine Center at the Richard Roudebush VA Medical Center and Director of the Center for Translational Sciences Institutes small animal model Cardiovascular Ischemia and Vasculogenesis Core. His academic research involves evaluating the mechanisms of action and translational potential of stem cell therapies for diseases characterized by vascular dysfunction. Dr. Johnstone is a founder of two other Indianapolis area start-up Biotech companies: one developing a small molecule drug for Lou Gehrig's disease; the other developing biological factors derived from stem cells for multiple neurological diseases, including stroke.

Previously he was an early employee of Sangamo BioSciences, located in the San Francisco Bay area. During Dr. Johnstone's six years at Sangamo he was responsible for running the preclinical development program for their lead therapeutic compound for cardiovascular diseases. His area of expertise in drug development is preclinical development through IND submission and has involved many direct interactions with regulatory authorities. As an active member of EmphyMab, he is directing the early development of a monoclonal antibody for the first therapeutic target of Chronic Obstructive Pulmonary Disease (COPD) resulting from emphysema. He is also engaged in strategic evaluation and selection of additional therapeutic indications as wells as establishing a pipeline of candidate molecular entities for addressing unmet medical needs in diseases of vascular inflammation and injury.

### Matthias Clauss, Ph.D.

Dr. Clauss received his PhD from the University of Heidelberg and his postdoctoral training from the Columbia University in New York. He is Associate Research Professor of Cellular and Integrative Physiology and member of the Indiana Center for Vascular Biology and Medicine at IU School of Medicine. His research program analyzes mechanisms of endothelial cell activation and aims to identify the links between inflammation and angiogenesis. A specific focus is on the regulation of angiogenic (VEGF) and anti-angiogenic (EMAPII) factors and their receptor-mediated signaling. In an ongoing collaboration with Dr. Petrache, who was the first to demonstrate the link between endothelial cell death and emphysema, EMAPII neutralizing antibodies were established as therapeutic tools for treating cigarette smoke induced emphysema. As EMAPII both induces apoptosis and is released during this process, it is their central hypothesis that EMAPII is part of a self-sustaining machinery of destruction in the progression of lung emphysema induced by cigarette smoking. As founding member of EmphyMab, Matthias Clauss is poised to help bringing this antibody forward to clinical application in addition to further analyzing the mechanism of how this antibody interferes with the induction and progression of emphysema.

### Irina Petrache, M.D.

Dr. Petrache is Professor of Medicine, Biochemistry, and Molecular Biology at the Indiana University. She is a physician scientist, board certified in Pulmonary and Critical Care Medicine, and principal investigator of a laboratory that studies mechanisms of lung injury and repair pertinent to cigarette smoke-induced chronic lung disease. The projects in the laboratory employ a translational approach, investigating intermolecular interactions, cell biology, animal models of disease, and human biological samples to answer questions related to the lung and systemic effects of cigarette smoking. The Petrache laboratory's main goal is to identify targets of therapy and to quickly bring them to bedside to help individuals who suffer from COPD such as emphysema. This interest led to Emphymab, Inc, where Dr. Petrache is involved as a founding member and a contributor to research directions and clinical insight.

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### Development Plans/Needs

- 1. Development and validation of a lead candidate high specificity, fully humanized EMAP II monoclonal antibody.
- 2. Following attract substantial funding to continue non-clinical development as well as potentially perform early safety and proof-of-concept clinical trials.
- 3. Explore opportunities to partner or out-license this program to a larger company possessing the necessary resources for late-stage development and commercialization.