



# BIKAM Pharmaceuticals

Therapeutics for Retinal Degenerative Disease

BIKAM Pharmaceuticals is an early-stage biopharmaceutical company dedicated to the development of novel therapeutics to treat retinal degenerative disease. The company is focused on the development of small molecule therapeutics based on a novel mechanism of action identified by the company's co-founder, Dr. Shalesh Kaushal.

## Technology

It is now clear that a large number of genetic diseases (such as Retinitis Pigmentosa) result from the improper folding of mutant proteins. Misfolded proteins often do not progress properly within the cell biosynthetic machinery and do not reach the appropriate site of action. This impairs the normal functioning of the cell. The effects of mounting levels of misfolded proteins within the cell can ultimately lead to cell death and tissue degeneration, resulting in disease. These misfolded proteins can sometimes find their correct location if supplied with a small molecule that can act as a catalyst to facilitate the folding and trafficking to the proper location within the cell. Small molecules in these circumstances are referred to as *pharmacologic chaperones*.

BIKAM is on the forefront of this emerging field and is seeking to discover a small molecule, non-retinoid pharmacological chaperone for Retinitis Pigmentosa (RP). RP is a progressive, orphan disease with a strong genetic background, and in many cases caused by the misfolding of mutant opsin, the protein that ultimately forms the visual pigment, rhodopsin. There are no real therapies to treat the disease, and essentially all RP sufferers will eventually go blind.

## Market Potential

Ophthalmic research is progressing at an accelerated rate due to the growing global demand for eye care and quality products to improve vision and enhance life. This is in part due to the aging of the worldwide population and the resulting increased rate of age related eye disease. Additionally, as surgical, cosmetic and augmentation approaches begin to plateau, the demand for new therapeutics will increase.

BIKAM's initial program is focused upon developing a treatment for RP. This is an orphan disease with approximately 110,000 people in the U.S. suffering from the disease. It occurs in about 1 in 3,000 people and is essentially genetically derived. The company's second program focuses on dry age related macular degeneration (dry AMD). There are approximately 1.5 million people with dry AMD in the US. There are currently no therapies for either RP or dry AMD.



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## Strategy

Scientifically, BIKAM has taken both a substrate and structure based approach to discover chemical starting points for the RP program. The substrate based approach uses the molecular architecture of the natural ligand as the starting point, while the structure based approach uses a computation model generated from the X-ray structure of bovine rhodopsin. Using these two medicinal chemistry approaches we have prepared a number of proprietary molecules that have been evaluated and progressed through a number of engineered cellular and animal models of the disease. We have shown, unequivocally, that we can discover small molecule pharmacologic chaperones with the potential to treat RP.

BIKAM's business strategy is to operate the company with a very lean infrastructure. We will outsource as many of the research and development activities as possible and seek to identify viable development candidates in each of our research indications. At that point, we will look to put collaborative agreements in place with either large or specialty pharmaceutical companies to further develop these assets.

## Management Team

### **Muzammil Mansuri, Ph.D. - CEO and President**

Dr. Mansuri has worked for BMS, Mitotix, GPC Biotech, Flagship Ventures, CGI Pharmaceuticals and Sequoia. He has a Ph.D. in organic chemistry.

### **David Garvey, Ph.D. - Vice President**

Dr. Garvey has worked for Abbott and NitroMed. He has a Ph.D. in chemistry.

### **Gregory LaRosa, Ph.D. - Vice President**

Dr. LaRosa has worked for Repligen, Leukocyte, Millennium, and Critical Therapeutics. He has a Ph.D. in cellular and developmental biology.

### **Sona Kaushal - Senior Director of Operations**

Ms. Kaushal has a B.S. in psychology. She coordinates the day-to-day activities in BIKAM's Florida labs.

## Contact Information

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