Visual Response Improvement for Individuals with Retinal Photoreceptor Degeneration  
(VA Reference No. 11-132)

**Administration of a GABA-C receptor antagonist which could potentially improve vision for age-related macular degeneration and retinitis pigmentosa**

<table>
<thead>
<tr>
<th>Technology</th>
<th>Therapy for retinal photoreceptor degeneration</th>
</tr>
</thead>
</table>
| Inventors  | Ralph Jensen, Ph.D.  
VA Boston Healthcare System |
| Key Features | • GABA receptor antagonist which can increase light sensitivity  
• Could potentially improve vision for age-related macular degeneration and retinitis pigmentosa  
• Neurological side effects are expected to be minimal or nonexistent  
• Attractive drug candidate in a market without a current product that can increase light sensitivity in patients |
| Stage of Development | Reduced to practice with demonstration in a rat model of retinitis pigmentosa |
| Keywords | Photoreceptor degeneration  
Vision improvement  
Age-related macular degeneration  
Retinitis pigmentosa  
GABA receptor |
| Patent Status | Provisional Patent |
| Contact | Adaku Nwachukwu, MS, JD  
Technology Transfer Program  
Department of Veterans Affairs  
Office of Research & Development (12TT)  
810 Vermont Avenue, NW  
Washington, DC 20420  
Phone: 202-443-5644  
adaku.nwachukwu@va.gov |

Research at the Department of Veterans Affairs (VA) resulted in the discovery that the GABA-C receptor antagonist (1,2,5,6-Tetrahydropyridin-4-yl)methylphosphinic acid (TPMPA) increased light sensitivity in a rat model of retinitis pigmentosa. Application of TPMPA caused retinal ganglion cells to increase light sensitivity, which corresponded to the cells responding to light that was 6 times less intense. The administration of TPMPA could potentially restore visual responses in individuals suffering from vision loss due to photoreceptor degeneration.

**Opportunity**

AMD is the most common cause of blindness in industrialized countries with 50 million people affected worldwide. Retinitis pigmentosa affects about 1 in 3,000 to 1 in 4,000 people in the world. Based on the prevalence of retinitis pigmentosa, the disease affects approximately 75,000 people in the United States and 1.75 million worldwide. Both of these diseases along with other visual disorders have a significant impact with the total financial cost of major visual disorders among United States residents 40 years or older estimated at over $35 billion. The global AMD therapeutic market alone is expected to grow to $3.0 billion by 2017.

**Competitive Advantage**

Currently there is not a method to increase light sensitivity in patients with a retinal disease that leads to a loss of photoreceptors. Therapy incorporating the delivery of TPMPA to the retina would have an advantage over existing therapy for AMD and retinitis pigmentosa. TPMPA could potentially lessen or alleviate the reduction in vision for these diseases. In addition, since GABA-C receptors are primarily in the retina, it is anticipated that neurological side effects would be minimal or nonexistent.

**Status**

The Department of Veterans Affairs is looking for a partner for further development and commercialization of this technology through a license and the VA inventors are available to collaborate with interested companies through a Cooperative Research and Development Agreement (CRADA).