

Gene Therapy Drug for Dry AMD Undergoes Phase I Clinical Trial

AMD is currently the leading cause of blindness and affects more than 6 million people in the U.S. and over 20 million worldwide. While effective therapies exist for the 15% of patients who have wet AMD, there is no FDA-approved treatment for the 85% of patients living with dry AMD. This may not be true for long.

A number of biotech and pharmaceutical companies have competing ideas on how to avoid or treat dry AMD. Late last year, Hemera Biosciences, of Massachusetts, was granted an IND (Investigational New Drug) to begin a small phase I clinical trial. The company is led by highly respected ophthalmologists at Tufts University.

So, how does the new drug work? Scientists have identified a cellular response – an overactive inflammatory cascade called “Membrane Attack Complex” (MAC) in the development of dry AMD. The accumulation of an overactive MAC on cell surfaces may lead to early cell death (apoptosis) which is suspected to be the leading cause of dry AMD and geographic atrophy (GA).

Researchers believe that in individuals with AMD, MAC develops faster than the cells can protect themselves, which leads to more cell deaths. The Hemera product is a gene therapy: a one-time dose of the drug will be injected directly into the eye by an ophthalmologist. This drug will cause normal retinal cells to increase expression of the soluble form of CD59 (sCD59), a factor that the body creates to protect retinal cells against MAC.

The result should preserve retinal cell life and avoid the development or progression of dry AMD.

The phase I study will enroll 25 patients with advanced AMD living in the Boston area. The study will test the safety of the injection and follow these patients for six months. Within this timeframe, investigators will determine the best dosage to achieve maximum effectiveness with the minimum of side effects. Like all clinical trials, these procedures are still considered to be experimental, but several professionals hold high hopes for this therapy as a treatment for dry AMD which affects the lives of so many seniors.