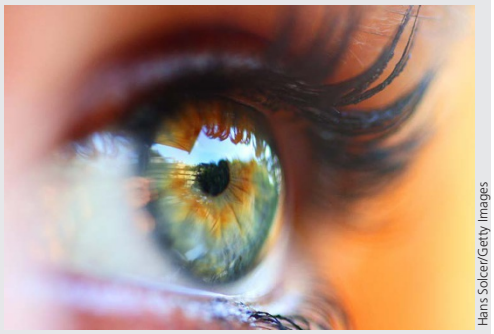
NEWS BRIEFS

Canadian Medical Association urges oversight of DTC testing

Right now, it's buyer beware in Canada, as direct-to-consumer (DTC) genetic test services proliferate with no real limits on marketing practices. In response, the Canadian Medical Association (CMA) has issued its first statement urging a multifaceted approach to protect DTC genetic test consumers. The CMA's policy recommendations cover consumer privacy protections, physician education, proposed government regulation, and industry accreditation. The organization of medical providers in Canada felt the need for the statement as online marketing of tests expands and companies offer tests with little or no guarantee of reliability or validity. A company that can't guarantee the reliability or validity of its tests should be prohibited from "making claims about the potential medical utility of its test and/or its potential to improve health," according to the policy statement. Further, the CMA recommends that companies be required to obtain informed consent that clearly states how data will be collected and used, who will have access to the data, what safeguards are in place to protect it, and how it will be disposed of in the event a company folds or is sold. The organization also recommends that physicians explain to patients the inherent limited reliability of DTC test results and, if concerns remain, to order follow-up testing from a certified, accredited medical laboratory. It calls on the Canadian government to "establish clear boundaries for the marketing, distribution, accreditation, and third-party use of DTC genetic tests." Such standards should be drafted to keep pace with the rapid development of genetic testing and should "hold companies accountable for being transparent about their uses of data/DNA and the potential resale of such material." *—Karyn Hede, News Editor*

ASSOCIATION MÉDICALE CANADIENNE | CANADIAN MEDICAL ASSOCIATION

Gene therapy for retinal disease receives orphan drug designation



Hans Solcer/Getty Images

An experimental gene-based treatment for inherited retinal disease has been accepted for review by the Food and Drug Administration, with a decision expected in early 2018. If approved, it would be the first corrective gene-based treatment approved for sale in the United States. The treatment, developed by Spark Therapeutics, Philadelphia, PA, had already received FDA orphan drug designation in July 2017. It would also be the first treatment available for vision loss caused by mutations in the *RPE65* gene. People who inherit biallelic *RPE65* gene mutations experience night blindness, involuntary eye movements, and tunnel vision that eventually progresses to total blindness. The results of a randomized, controlled phase III clinical trial of the investigational treatment, voretigene neparvovec, published in *The Lancet* on 13 July 2017, showed meaningful gains in vision maintained over at least two years. Crucially, no patients experienced the serious adverse events or immunological reactions that have plagued previous clinical trials that employed gene-based treatment administered via viral vector. That makes this trial, which included 31 patients, the first successful gene therapy trial in the United States. The company followed up that success with a similarly encouraging report in August, stating that it had achieved clinically meaningful results in a handful of patients treated with its gene therapy for hemophilia A. With hundreds of gene therapy trials planned or under way, hopes for further successes are currently riding high. *—Karyn Hede, News Editor*