The Focal Point: Advocacy & Legislative Update

February 4, 2020

In This Issue

EBAA Releases 2019-nCoV Screening Recommendations

EBAA released the 2019-nCoV screening recommendations for member eye banks [with precautionary donor deferrals for the protection of eye bank recovery staff and the safety of corneal tissue. These recommendations are as follows:

2019-nCoV Screening Recommendations for EBAA Member Eye Banks

Effective immediately, the EBAA recommends that eye banks exclude/defer (rule out) potential donors for ocular tissue that in the last 28 days before donation met one or more of the following criteria:

- Travel to mainland China (regardless of symptoms);

- Travel to other geographic area designated as area of active transmission by the CDC (*https://www.cdc.gov/coronavirus/2019-ncov/index.html*) **WITH**
    - presentation of symptoms consistent with 2019-nCoV (e.g., unexplained fever, cough, shortness of breath) **OR**
    - exposure link to suspected case patient while in the designated area
- Close contact with a person who has confirmed 2019-nCoV infection, including healthcare workers
- Test positive for 2019-nCoV
- Symptoms consistent with active 2019-nCoV infection (e.g., unexplained fever, cough, shortness of breath) in a patient with suspected 2019-nCoV infection

This will be in effect until further notice or additional criteria are added. Screening recommendations shall expire when 2019-nCoV is no longer endemic.

Situation report as of February 3, 2020:

- Globally there have been 20,680 confirmed cases (427 deaths) of the novel Coronavirus (2019-nCoV). The vast majority of the cases are inside China; about 163 cases have been confirmed in at least 24 other countries.
- The United States currently has 11 confirmed cases of 2019-nCoV; including 2 cases of person-to-person spread of the virus among spouses.
- The first death has been reported outside of China, in the Philippines, in a patient who was a close contact of the first confirmed case in the Philippines. Hong Kong reported its first death on Feb 4.
- The U.S. has declared a Public Health Emergency, denying entry to foreign nationals who had recently visited China and imposing 14-day quarantines on American citizens returning from mainland China.

FDA Releases Draft CJD and vCJD Guidance for Blood Establishments

The FDA issued a draft guidance document to provide blood with revised recommendations intended to reduce the possible risk of transmission of Creutzfeldt-Jakob disease (CJD) and variant Creutzfeldt-Jakob disease (vCJD) by blood and blood components. The recommendations apply to the collection of whole blood and blood components intended for transfusion or for use in further manufacturing, including source plasma and **do not apply to HCT/Ps at this time**.

The new recommendations in the draft guidance narrow the geographic deferral to the United Kingdom, France and Ireland. The draft guidance also removes deferral for the following: time spent on United States military bases in Europe; receipt of a blood transfusion in certain variant Creutzfeldt-Jakob Disease (vCJD) risk countries; risk factors for iatrogenic Creutzfeldt-Jakob Disease (CJD) (i.e., a history of taking human cadaveric pituitary-derived growth hormone); having blood relatives with CJD; and a history of injecting bovine insulin.

This draft guidance is for comment purposes only, and implementation is not recommended at this time. When finalized, the guidance will supersede the January 2016 guidance, "Revised Preventive Measures to Reduce the Possible Risk of Transmission of Creutzfeldt-Jakob Disease and Variant Creutzfeldt-Jakob Disease by Blood and Blood Products, Guidance for Industry."

Although this guidance does not apply to tissue establishments, EBAA has actively advocated for revised donor deferral recommendations for many years, so eye banks may wish to submit comments to FDA. Comments should be submitted by March 31st.

Cardinal Health Voluntarily Recalls Procedure Packs Containing Contaminated Surgical Gowns

Cardinal Health voluntarily recalled more than 2.9 million procedure packs manufactured between September 2018 and January 2020 that contain potentially contaminated surgical gowns.

This recall comes about a week after Cardinal recalled 9.1 million surgical gowns that may have been exposed to bacteria and other contaminants at unauthorized manufacturing sites in China, which may have infected patients. While Cardinal sterilizes the products after they are manufactured, it could not verify that they were sterile because it could not quantify their exposure to bacteria.

The supplies in 2.5 million packs were not individually separated by sealed packages, while 374,794 packs had individually sealed components in the "sterilization pouch." Cardinal instructed customers to discard all other components in those packs.

Healthcare facilities are scrambling to find alternatives and eye banks should brace for long-term effects to their supply chains. EBAA has a preferred relationship with Medline to support our member banks for their lab supplies, including surgical gowns and face masks. Medline will prioritize their allocation to current customers and new customers who sign a “Commitment Agreement.”

AOPO Announces Steve Miller as New Chief Executive Officer

The Association of Organ Procurement Organizations (AOPO), announced that Steve Miller will join the organization as its new Chief Executive Officer (CEO) on February 17. As CEO, Miller will lead AOPO in its mission to work with the 58 federally-designated member Organ Procurement Organizations (OPOs) across the United States to maximize the availability of organs and tissues for transplantation, and enhance the quality, effectiveness and integrity of the donation process.

Miller most recently served as the Chief Operating Officer (COO) at the Ambulatory Surgery Center Association (ASCA). Prior to serving as COO, Miller was their Director of Government and Public Affairs. Miller's career includes positions at the American Academy of Ophthalmology, American Health Care Association, the Office of Representative James Bilbray and the Office of Senator Harry Reid.

FDA Finalizes Six Gene Therapy Guidance and Unveils a New Draft

The FDA has finalized six guidance documents on gene therapy manufacturing and clinical development, and has released a new draft guidance on interpreting the sameness of gene therapies under the orphan drug regulations.

The six final guidance documents finalize drafts from July 2018 and focus on developing hemophilia, rare disease and retinal disorder gene therapies, and include one on chemistry, manufacturing and control (CMC) information, one on long term follow-up observational studies collecting data on adverse events and one on the testing of retroviral vector-based therapies.

Interpreting Sameness of Gene Therapy Products Under the Orphan Drug Regulations

The new draft guidance on sameness explains how FDA will decide if orphan exclusivity will be awarded if two gene therapy products are intended for the same use or indication.

When assessing two treatments, FDA will consider both the therapeutic gene and the inactivated virus used to deliver it. A treatment relying on a different viral vector, for instance, would be considered by the FDA as a different product and eligible for orphan drug exclusivity.

The FDA said it would assess differences between vectors from the same viral family on a "case-by-case" basis.

Human Gene Therapy for Retinal Disorders

This guidance focuses on issues specific to GT products for retinal disorders and provides recommendations related to product development, preclinical testing, and clinical trial design for such GT products. This guidance finalizes the draft guidance of the same title dated July 2018.

This final version remains largely similar to the draft, with some new details on safety considerations and further explanation of why a single administration of a gene therapy (GT) product in each eye may not always be sufficient.

FDA recommends multiple measures to reduce bias including at least two treatment arms, utilizing different doses but the same product administration procedures, and separation of clinical evaluators and personnel involved in product administration/sham procedure to minimize patients' ability to identify their treatment arm, in addition to a sham control group.

- Human Gene Therapy for Retinal Disorders
- Human Gene Therapy for Hemophilia
- Human Gene Therapy for Rare Diseases
- Chemistry, Manufacturing, and Control (CMC) Information for Human Gene Therapy Investigational New Drug Applications (INDs)
- Long Term Follow-up After Administration of Human Gene Therapy Products
- Testing of Retroviral Vector-Based Human Gene Therapy Products for Replication Competent Retrovirus During Product Manufacture and Patient Follow-up
- Testing of Retroviral Vector-Based Human Gene Therapy Products for Replication Competent
- Retrovirus During Product Manufacture and Patient Follow-up

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CDER Unveils List of 2020 Guidance Documents

The FDA, Center for Drug Evaluation and Research (CDER) unveiled its list for 2020 guidance documents, with the bulk of the new and revised drafts falling into categories related to generic drugs, pharmaceutical quality/CMC and procedural work.

Several proposed guidance which may be of interest, are:

- Quality Considerations for Topical Ophthalmic Drug Products
- Quality and Stability Testing of Drug Substances and Drug Products for NDAs, ANDAs, and BLAs and Associated Labeling Statements for Drug Products
- Microbiological Quality Considerations in Non-Sterile Drug Product Manufacturing
- Promotional Labeling and Advertising Considerations for Prescription Biological Reference and Biosimilar Products--Questions and Answers

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Judge Rules Against FDA in Bid to Shut Down California Stem Cell Clinics

A federal judge denied a motion for summary judgment filed by the Department of Justice (DOJ) in a lawsuit against two California stem cell clinics. DOJ, on behalf of FDA, filed the injunction to shut down the California Stem Cell Treatment Center and the Cell Surgical Network for using unapproved drugs to treat patients suffering from several chronic and degenerative conditions.

In his ruling, Judge Jesus G. Bernal cited a recent Supreme Court decision in finding that FDA's interpretation of its rules defining stem cell preparations as illicit drugs "is not entitled to deference." The Supreme Court case, Kisor v. Wilkie, narrowed the circumstances in which courts must defer to a federal agency's interpretation of its own rules. Bernal ruled that the case was suitable for trial.

FDA OKs First Drug for Thyroid Eye Disease

The FDA approved teprotumumab (Tepezza) for adults with active thyroid eye disease, making it the only non-surgical, FDA-approved treatment for this potentially blinding condition.

Teprotumumab is a fully human monoclonal antibody inhibitor of insulin-like growth factor-1 receptor and is administered in eight doses, given as 30- to 60-minute infusion once every 3 weeks. The FDA greenlight was not a surprise, after an FDA advisory committee unanimously voted (12-0) in favor of teprotumumab's approval last month.

Thyroid eye disease is an autoimmune condition where antibodies inflame muscles behind the eye, causing outward bulging of the eye that can cause a variety of symptoms such as eye pain, double vision, light sensitivity or difficulty closing the eye. This disease impacts a relatively small number of Americans, but the condition can be incapacitating.

RESEARCH NEWS

CorNeat Vision Develops Easily Implantable Artificial Cornea

CorNeat Vision, based in Israel is developing an easily-implantable and affordable artificial cornea that could one day replace donor corneas in people needing transplants and therefore address the shortage of donor tissue.

The synthetic cornea medical device needs less suturing and is designed to integrate better with fibroblast cells to seal the implant into the front of the eye.

The implant has shown promise for restoring the vision of rabbits after implantation. CorNeat is now applying for clearance to enter phase I trials in humans and hopes to get market approval for the synthetic cornea in 2021.

Medical Gel Prepares to Enter Clinical Trials for Replacing Corneal Transplants

An international team of researchers in Europe, Asia, and North America is developing a liquid gel that sets at body temperature and which can be modified to act as a tissue glue. This “liquid cornea” material seals corneal perforations and prevents the need for any subsequent transplants. If successful, the biosynthetic could be applied through a syringe within 30 minutes, without the need for a donor cornea and invasive surgery

According to the Tej Kohli Foundation, an Indian non-profit leading the project, the gel could enter clinical trials within three years.